



Solid Biosciences to Provide Update on Long-Term Outcomes Data from Patients in the Ongoing IGNITE DMD Phase I/II Clinical Trial of SGT-001

September 20, 2021

- Functional and patient-reported outcomes data for the first three patients in the high-dose (2E14 vg/kg) cohort (Patients 4-6) 1.5 years post-dosing to be presented at the World Muscle Society 2021 Virtual Congress -

CAMBRIDGE, Mass., Sept. 20, 2021 (GLOBE NEWSWIRE) -- Solid Biosciences Inc. (Nasdaq: SLDB), a life sciences company focused on advancing meaningful therapies for Duchenne muscular dystrophy (Duchenne), today announced the presentation of long-term data from Patients 4-6 in the ongoing IGNITE DMD Phase I/II clinical trial of SGT-001 in patients with Duchenne. Vamshi Rao, MD, Attending Physician, Neurology, Lurie Children's Hospital of Chicago and Assistant Professor of Pediatrics (Neurology and Epilepsy), Northwestern University Feinberg School of Medicine, and an IGNITE DMD clinical investigator, will present the data on September 23, 2021 in an oral session at the World Muscle Society 2021 Virtual Congress, which is taking place September 20-24.

Presentation Details

Title: IGNITE DMD Phase I/II ascending dose study of SGT-001 microdystrophin gene therapy for DMD: Update on long-term outcomes

Presenter: Dr. Vamshi Rao

Presentation Type: Oral presentation

Abstract Number: O.2

Presentation Time: September 23, 2021, 9:00am ET

The presentation will be available to individuals registered to attend the World Muscle Society 2021 Virtual Congress. To register for the conference, please visit: <https://www.wms2021.com/page/registration>

About SGT-001

Solid's SGT-001 is a novel adeno-associated viral (AAV) vector-mediated gene transfer therapy designed to address the underlying genetic cause of Duchenne. Duchenne is caused by mutations in the dystrophin gene that result in the absence or near absence of dystrophin protein. SGT-001 is a systemically administered candidate that delivers a synthetic dystrophin gene, called microdystrophin, to the body. This microdystrophin encodes for a functional protein surrogate that is expressed in muscles and stabilizes essential associated proteins, including neuronal nitric oxide synthase (nNOS). Data from Solid's clinical program suggests that SGT-001 has the potential to slow or stop the progression of Duchenne, regardless of genetic mutation or disease stage.

SGT-001 is based on pioneering research in dystrophin biology by Dr. Jeffrey Chamberlain of the University of Washington and Dr. Dongsheng Duan of the University of Missouri. SGT-001 has been granted Rare Pediatric Disease Designation, or RPDD, and Fast Track Designation in the United States and Orphan Drug Designations in both the United States and European Union.

About Solid Biosciences

Solid Biosciences is a life sciences company focused on advancing transformative treatments to improve the lives of patients living with Duchenne. Disease-focused and founded by a family directly impacted by Duchenne, our mandate is simple yet comprehensive – work to address the disease at its core by correcting the underlying mutation that causes Duchenne with our lead gene therapy candidate, SGT-001. For more information, please visit www.solidbio.com.

Forward-Looking Statements

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements regarding the ability of the Company to continue dosing patients in the IGNITE DMD trial, the implication of interim clinical data, the safety or potential treatment benefits of SGT-001 in patients with DMD, the Company's expectations for reporting future data from the IGNITE DMD trial, the Company's regulatory plans and timelines and other statements containing the words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "would," "working" and similar expressions. Any forward-looking statements are based on management's current expectations of future events and are subject to a number of risks and uncertainties that could cause actual results to differ materially and adversely from those set forth in, or implied by, such forward-looking statements. These risks and uncertainties include, but are not limited to, risks associated with the Company's ability to or continue IGNITE DMD on the timeline expected or at all; obtain and maintain necessary approvals from the FDA and other regulatory authorities; obtain and maintain the necessary approvals from investigational review boards at IGNITE DMD clinical trial sites and the IGNITE DMD independent data safety monitoring board; enroll patients in IGNITE DMD on the timeline expected; the Company's dosing strategy; replicate in clinical trials positive results found in preclinical studies and earlier stages of clinical development; whether the interim data presented in this release will be predictive of the final results of the trial or will demonstrate a safe or effective treatment benefit of SGT-001; whether the methodologies, assumptions and applications we utilize to assess particular safety or efficacy parameters will yield meaningful statistical results; advance the development of its product candidates under the timelines it anticipates in current and future clinical trials; successfully optimize and scale its manufacturing process; obtain, maintain or protect intellectual property rights related to its product

candidates; compete successfully with other companies that are seeking to develop Duchenne treatments and gene therapies; manage expenses; and raise the substantial additional capital needed, on the timeline necessary, to continue development of SGT-001, achieve its other business objectives and continue as a going concern. For a discussion of other risks and uncertainties, and other important factors, any of which could cause the Company's actual results to differ from those contained in the forward-looking statements, see the "Risk Factors" section, as well as discussions of potential risks, uncertainties and other important factors, in the Company's most recent filings with the Securities and Exchange Commission. In addition, the forward-looking statements included in this press release represent the Company's views as of the date hereof and should not be relied upon as representing the Company's views as of any date subsequent to the date hereof. The Company anticipates that subsequent events and developments will cause the Company's views to change. However, while the Company may elect to update these forward-looking statements at some point in the future, the Company specifically disclaims any obligation to do so.

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